

**IMPROVED RAAV VECTORS FOR ENHANCING TRANSDUCTION OF CELLS
EXPRESSING LOW-DENSITY LIPOPROTEIN RECEPTORS**

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ABSTRACT

Disclosed are methods for the use of therapeutic polypeptide-encoding polynucleotides in the creation of transformed host cells and transgenic animals. In particular, the use of recombinant adeno-associated viral (rAAV) vector compositions that specifically target mammalian cells, such as pancreatic islets cells, that express low-density lipoprotein receptors on their cell surface. The disclosed vectors comprise one or more polynucleotide sequences that express one or more mammalian polypeptides having therapeutic efficacy in the amelioration, treatment and/or prevention of AAT- or cytokine polypeptide deficiencies, such as for example in diabetes and related diseases, as well as a variety of autoimmune disorders including, for example, lupus and rheumatoid arthritis.

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